Written Submission for Health Canada's National Strategy for High-Cost Drugs for Rare Diseases Online Engagement

By: ALS Society of Canada

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INTRODUCTION:

On behalf of the amyotrophic lateral sclerosis (ALS) community in Canada, the following is the ALS Society of Canada (ALS Canada)'s written submission providing feedback on the *Building a National Strategy for High-Cost Drugs for Rare Diseases* discussion paper.

ALS is a terminal disease of progressive paralysis. In Canada, approximately 1,000 people die of it each year. This is a disease that can move with startling swiftness: four out of five people die within two to five years of their diagnosis. With several ALS therapies in later-stage clinical trials, the status quo of the drug access pathway does not respond to the urgent reality of ALS: in the time it takes for a therapy to move through regulatory approval to the reimbursement decisions that result in patient access, thousands of Canadians will die waiting to access the ALS treatments they need.

Governments in Canada have recognized issues surrounding access to therapies for rare diseases like ALS. However, each consultation, review or suggested solution is typically built around cost containment, and not focused on access and innovation. Governments do not tend to include the cost to society of a person out of work, additional public health care costs and more that could be mitigated or solved by a new medication – let alone the quality of life for the person living with ALS and their family. In the end, this approach could affect the ability of the ALS community to access proven ALS therapies as quickly as they need them.

While we appreciate the opportunity to provide input into this Health Canada process, the discussion paper itself created challenges for meaningful feedback. The process for how drugs are accessed and paid for in Canada is complex and requires a baseline of technical knowledge to understand. Further, from the outset we found elements of bias obvious throughout the paper. Simply the use of the term "high-cost" has a clearly negative connotation, suggesting that anyone who requires access to these medications is somehow at fault for the financial pressures inherent in our health system. We reject this premise. People living with ALS and other disorders considered to be rare desperately need access to medications that may help them to live and to maintain quality of life, in the face of often devastating diseases that are not of their own making. We should not lose sight of their lived reality.

Having said that, we also found elements of the paper to be thoughtful and robust in content.
And so, to bring forward the perspectives and insight of people affected by ALS, and help government understand how the options put forward in the discussion paper could impact this vulnerable community, we surveyed the ALS community in Canada using the issue/question format in the discussion paper. The survey ran from March 8 to 22, 2021. A summary of the **246 responses received** is provided in each section below.

Given the ALS community faces issues related to access to therapies that are outside of the parameters of this consultation, we will seek opportunities for an ongoing dialogue and will be developing a position paper for publication in summer 2021 to provide a more holistic perspective on the challenges and opportunities associated with timely, equitable and affordable access to ALS therapies.

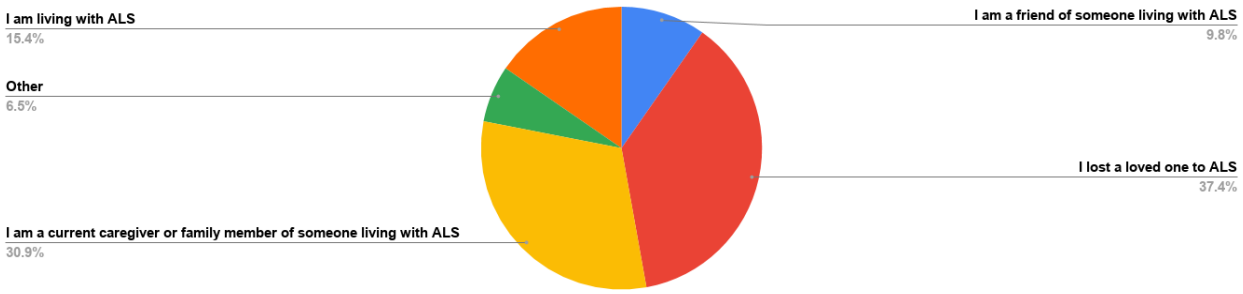
GEOGRAPHY:

The geographic make-up of the respondents was as follows:

AB	4.9%	NS	1.6%	PE	0
BC	9.8%	NT	0	QC	7.3%
MB	2.4%	NU	0	SK	1.2%
NB	0.4%	ON	72%	YT	0
NL	0.4%				

PERSPECTIVES:

People currently affected by ALS were in the majority of survey respondents: current caregivers or family members comprised 30.9% of respondents; people living with ALS comprised 15.4% of respondents; and friends of people living with ALS represented 9.8% of respondents. People who have lost a loved one to ALS represented 37.4% of respondents. The remaining 6.5% of respondents included people with professional expertise in ALS and family members or friends who have been devastated by the hereditary (or familial) form of ALS.



ISSUE 1:

"How to improve patient access to high-cost drugs for rare diseases and ensure that access is consistent across the country."

Each day, three Canadians are diagnosed with ALS and three will die from the disease. The community of approximately 3,000 Canadians living with the disease face unique challenges, with access to medications – from clinical trials through to reimbursement – being a significant one.

All of the proposed options for improving both timely access and national consistency for ALS treatments are relevant and it is critical that patients and clinicians continue to have an enhanced role within the current drug review processes. In addition, the creation of any principles or guidelines for assessing the value and effectiveness of therapies must include the patient voice, reflect the reality of rare diseases and consider the broad impact of a therapy on quality of life – especially from the perspective of the person living with the disease. That is to say, cost containment should not be the sole lens through which timely access and national consistency are considered.

ALS Canada Survey Responses
The following summarizes the responses from the ALS community related to Issue 1:

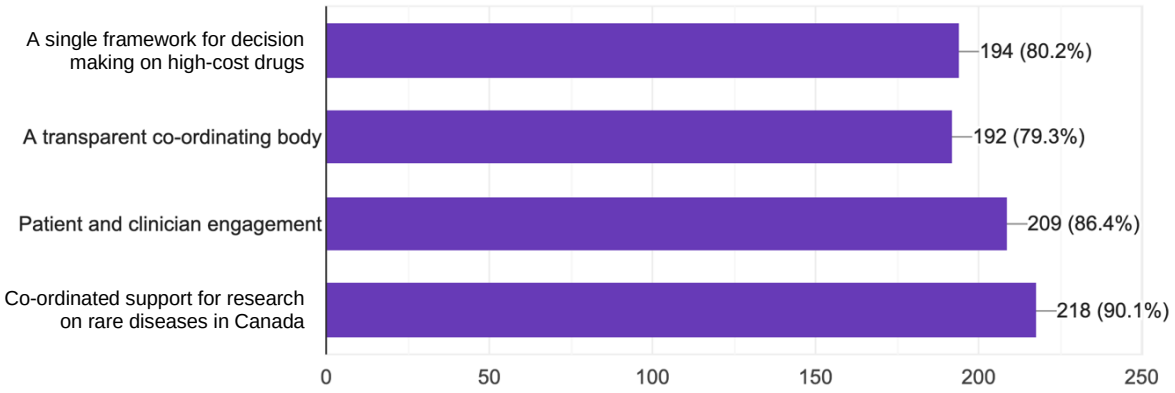
Q1. Is it important to you that the ALS community has timely access to all Health Canada approved medications to treat the disease?

- 100% of the respondents answered YES to Q1.

Q2. Is it important to you that the ALS community has consistent access across the country to all Health Canada approved medications to treat the disease?

- 98.8% of the respondents answered YES to Q2.
- 1.2% of the respondents answered NO to Q2.

Q3. Do you believe that any of the proposed options, or combination of options, would be effective at improving access and improving national consistency for ALS treatments?
(Select all that apply)



Q4. Are there any additional options, not included above, that you would like to suggest? Please explain.

Below is a summary of the responses received:

- People living with ALS should be given quick access to all medications that could potentially be beneficial:
  - based on lenient eligibility criteria
  - based on a 'scaling system' – i.e., a person with rapidly progressing disease would get priority
  - once approved in other parts of the world
  - even if only experimental/promising
  - through greater fast tracking at Health Canada
  - through a liaison agency that would work with patients/families/clinicians to access drugs
- Easy/quick access to drug trials
- Canada needs to make significant investments in research for rare diseases
- Accelerated reimbursement time with private insurance companies

Q5. Please provide any additional comments about the importance of timely and consistent access to medications for the ALS community.

Below is a summary of the responses received, along with some select quotes:

- With a diagnosis of ALS, there is typically no time to wait for access to medications:
  - "Our entire family may succumb to this disease. I don't know of any disease that affects the mortality of an entire family like familial ALS."
  - "It's a disease that evolves quickly so we cannot lose months on bureaucracy."
  - Many others noted that starting treatment early is key to ideally delaying the progression of ALS
  - "The very fast progression of ALS requires immediate and complete access to treatment at the time of diagnosis."

- “Equitable access and funding for medications which could slow the progression of the disease should be in place for all Canadians - not those who can afford to pay out of pocket.”
- “ALS patients in rural areas should have the same equal access to treatments and medications as urban ALS patients.”

ISSUE 2:

“How to ensure decisions on covering high-cost drugs for rare diseases are informed by the best evidence available.”

Unlike many rare diseases, reasonably sized trials with a good quantity of data collected are achievable in ALS, but these often remain smaller than trials for many other more prevalent conditions. As a result, the evidence may appear limited when compared to trials for other diseases. This ‘limited evidence’ should not delay or prevent access to ALS therapies, however it must be qualified through peer review.

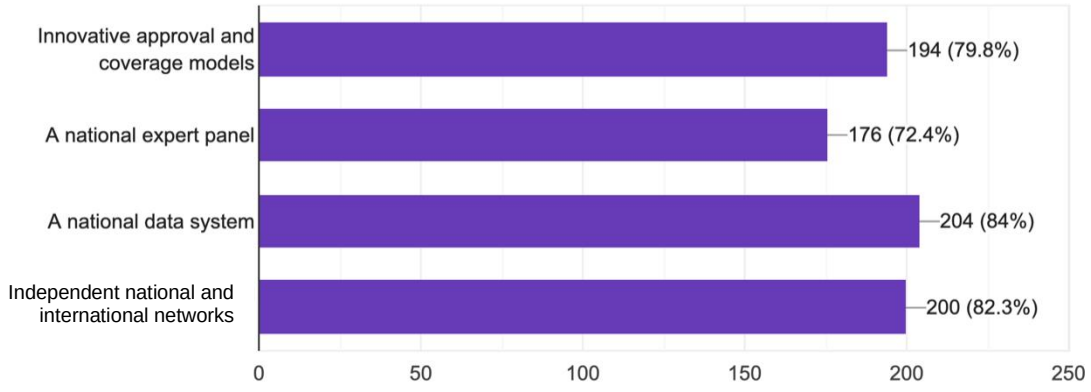
With respect to the innovative approval and coverage models option outlined in the discussion paper, objective indicators for measuring benefit could be challenging to develop for ALS. The symptom onset and rate of progression of ALS can be different from one person to another, and objective biomarkers and strong measures of efficacy are still being developed. As such, the benefits of a potential ALS therapy as reported through clinical trial data may seem insignificant to those without in-depth knowledge of the disease and understanding that even modest benefits can have a meaningful impact on quality of life. Any measures would clearly need to take into consideration the impact of benefits on patient quality of life. For example, the ability to move just one finger in one hand can mean the difference between independence and reliance because someone living with ALS can continue to operate the joystick on their wheelchair and a mouse on their computer. And without objective biomarkers and strong measures of efficacy in ALS, it's also challenging to determine specific outcomes for patients to be measured against.

ALS Canada Survey Responses
The following summarizes the responses from the ALS community related to Issue 2:

Q1. Should limited evidence delay or prevent access to ALS medications in Canada?

- 93.3% of the respondents answered NO to Q1.
- 6.7% of the respondents answered YES to Q1.

Q2. Do you believe that any of the proposed options, or combination of options, would be effective at strengthening the evidence base for ALS treatments? (Select all that apply)



Q3. Are there any additional options, not included above, that you would like to suggest? Please explain.

Below is a summary of the responses received, along with some select quotes:

- Caregivers should be included in any independent national and international networks
- A national expert panel must include clinicians with experience treating ALS, as well as equal patient representation from the community
    - "You do not need another committee or governing agency to add to the existing delays."
    - "I think a coverage model should simply include a diagnosis of ALS and give the specialist/neurologist the freedom to decide what to try. EVERY case is different."
    - "I believe Canada needs to be open to world studies for an ALS cure."

Q4. Please provide any additional comments about why limited evidence should not delay or prevent access to ALS medications in Canada.

Below is a summary of the responses received, along with some select quotes:

- Given the terminal nature of ALS and the typical lifespan after diagnosis, access to medications should not be delayed or prevented because of limited evidence – waiting for the full evidence will be too late for those diagnosed with ALS
    - "They don't have the time to wait for lengthy studies."
- Until there is research to support delaying access to ALS treatments, those treatments should be accessible
    - "People should be allowed to make informed decisions about their care/treatment."
    - "ALS patients urgently need access to any medications. They need all the hope they can get."
    - "ALS is 100% fatal. Some evidence is enough to try to help."
- Limited evidence does not necessarily mean there is limited effectiveness in treatments, as some ALS patients may still benefit from them

ISSUE 3:

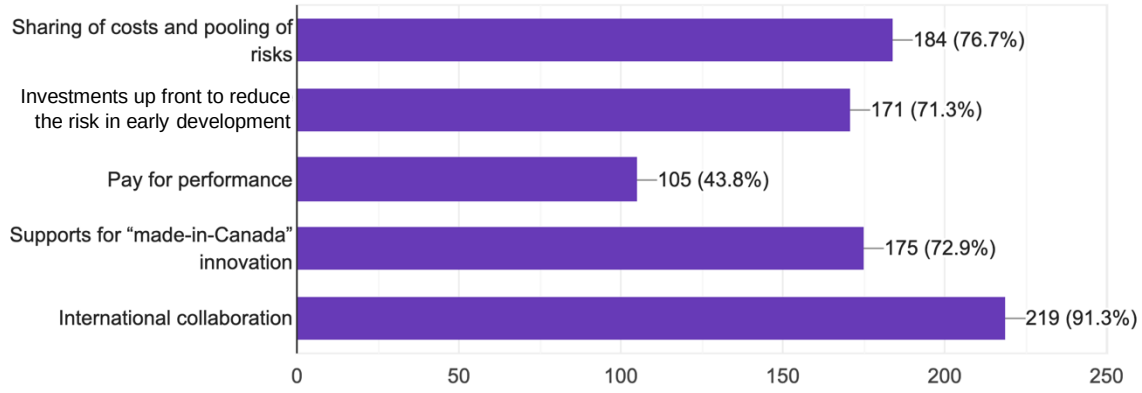
"How to ensure spending on high-cost drugs for rare diseases does not put pressure on the sustainability of the Canadian health care system."

There is no question that a sustainable healthcare system is of paramount importance. However, the cost of therapies is only one expense associated with healthcare. The way this issue is framed suggests an expectation that the cost of rare disease drugs should be reduced to the exclusion of all other opportunities for cost savings. The inherent message being sent is that Canadians unfortunate enough to be living with a rare disease are not deserving of therapies. When costs are factored in, a therapy could even lessen the disease cost in other ways (e.g. by offsetting costs to acute care, reducing caregiver burden). When considering opportunities to reduce expenses associated with healthcare, a more holistic view is desirable. This holistic view should also take into account indirect treatment costs (e.g., drugs that must be delivered via IV infusion).

The notion of a pay-for-performance model would be challenging for ALS therapies because of the disease's heterogeneity and subjectivity associated with quality-of-life improvements whereby individuals without in-depth knowledge of the disease may dismiss quality of life considerations that are particularly meaningful to someone living with the disease.

ALS Canada Survey Responses
The following summarizes the responses from the ALS community related to Issue 3:

Q1. Do you believe that any of the proposed options, or combination of options, would be effective for getting treatments for rare diseases, like ALS, to patients? (Select all that apply)



Q2. Are there any additional options, not included above, that you would like to suggest? Please explain.

Below is a summary of the responses received, along with some select quotes:

- The framing of this issue is "one dimensional" – there needs to be a more holistic understanding of the cost of failure to treat rare diseases and the needs of the patient
- Suggesting that the cost of drugs for people with rare diseases was putting an undue burden on the health care system "doesn't make sense" given the cost of treating much more common ailments – people with rare diseases are not "less worthy" of therapies
    - "Of course treatment for rare diseases will cost. More money is needed for research in Canada and collaboration with other worldwide research centres."
    - "Rare diseases are different than other diseases. It is difficult to develop and access treatments because of the small pool of potential benefits. As a result, I believe the general Canadian heath care system should provide some support to access treatments."
    - "By definition, any added cost will put strain on a budget since budgets are somewhat fixed. The real question is, what degree of strain is acceptable?"

ADDITIONAL QUESTIONS:

In addition to asking the ALS community to respond directly to the questions posed in the Discussion Paper, we sought feedback on building a strategy that will work in the context of Canada's health system and respect the role of provinces and territories in health care delivery.

ALS Canada Survey Responses
The following summarizes the responses from the ALS community related to the additional questions:

Q1. Do you agree that part of the successful implementation of a rare disease strategy for drugs (including ALS) in Canada would require the federal government to provide funding through transfer payments to the provinces/territories for the delivery of these medications?

- 96.2% of the respondents answered YES to Q1.
- 3.8% of the respondents answered NO to Q1.

Q2. Do you agree that part of the successful implementation of a rare disease strategy for drugs (including ALS) in Canada would require the provincial/territorial governments to ensure timely and consistent delivery of these medications with the federal transfer payments provided?

- 98.3% of the respondents answered YES to Q2.
- 1.7% of the respondents answered NO to Q2.

Q3. As a member of the ALS community, what else do you think should be considered as part of a national rare disease strategy, beyond the suggestions put forth in the discussion paper?

Below are some select quotes received:

- "Continue to work together to accelerate the drug approval process within the context of promising clinical trial drugs and with government/private funding."
- "I think collaboration and collecting data from ALS patients into one worldwide database is very important to finding a cure."
- "I think you overlooked the issue of assistive devices for mobility, communications, activities of daily living, etc."
- "More genetic testing and messaging about the incredible medical advances in treatments in order to eliminate the stigma and encourage those who have a family history to get tested."
- "I think there should be a category of diseases in this country, the ultra-terminal, that allows for faster access to drugs that may or may not yet be approved in Canada, and that allows the freedom of health care providers to try whatever they can, fully funded. Anything less is too slow, and costs people who NEED treatment their mobility, independence, and ultimately their lives."
- "There needs to be more money set aside for people having to care for someone with ALS and for the people living with ALS."
- "I think that a national drug strategy is a good thing. Drugs should not be denied to anyone who has a rare disease."